



Small Business Assistance

Financial Assistance and Incentives for Research and Development of New Drug/Biologic Products

**Ron Wilson, Director of Small Business Assistance
Center for Drug Development and Research, FDA
Ronald.Wilson@fda.hhs.gov**



Financial Assistance Pre-Approval and Financial Incentives Post-Approval

Financial Assistance Pre-Approval

- **Orphan Drug Program**
- **PDUFA**
- **Charging for Investigational Drugs**
- **Grants**

Financial Incentives Post-Approval

- **Orphan Drug Products Exclusivity**
- **New Chemical Entity Exclusivity (Hatch/Waxman)**
- **New Clinical Investigation Exclusivity (Hatch/Waxman)**
- **Pediatric Exclusivity**
- **180-Day Generic Drug Exclusivity**
- **Patent Term Extension**

Financial Assistance Pre-Approval

- **Orphan Drug Products**
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Orphan Drug Program

Orphan Drug Program

- **Identifies orphan drug products**
- **Facilitates their development in the treatment of rare diseases* and conditions**

****Rare disease means any disease or condition which affects less than 200,000 persons in the U.S***

Orphan Drug Program

Assistance Provided for Orphan Drug Designation

- Tax credits
- Research grants
- Waiver of PDUFA application and supplement fees

Prescription Drug User Fee Act (PDUFA)

- **Enacted in 1992**
- **Authorizes FDA to collect fees from companies that produce certain human drug and biological products**



PDUFA

- **Fees for drugs and biologics**
 - **Three types of users fees--Application Fees, Establishment Fees, and Product Fees**
 - **PDUFA Q&A for small businesses**
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PDUFA

Financial Provisions of PDUFA

- **Waivers**
 - **Reductions**
 - **Refunds**
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PDUFA

Waivers

- **Waiver of the application fee for the first human drug application that a small business (500 or less employees) or its affiliate submits for review is waived**
- **Waiver may be granted to any business for one or more fees**
- **Waiver may be granted if the assessment of the user fees would present a significant barrier to innovation due to limited resources or circumstances**



PDUFA

Reductions/Refunds

Reduction or refund of fees may be granted for an application or supplement that is refused for filing or withdrawn before or after filing





PDUFA

Procedures for Requesting Waiver, Reduction, or Refund of Fee

- To qualify, a written request must be submitted not later than 180 days after the fee is due
 - See PDUFA Q&A for specific information on how/where to submit a request
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Charging for Investigational Drugs

- **21CFR 312.7(d) permits sale of an investigational drug or biological product**
- **Certain requirements have to be met before charging is permitted**



Grants

- **Solicited grant applications**
 - **Unsolicited grant applications**
 - **Small Innovation Research Program**
 - **Small Business Technology Program**
 - **Grants & Funding Opportunities at NIH**
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Grants

Solicited Grant Applications

- **FDA solicits competitive applications through requests for applications (RFA)**
 - **RFA's are published in the federal register and other appropriate publications.**
 - **A list of RFA's can be found on the FDA grants opportunities page**
 - **RFA's are reviewed at NIH**
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Grants

Unsolicited Grant Applications

- **Unsolicited grant applications are those not submitted in response to a published request for application (RFA)**
 - **All unsolicited applications seeking FDA support should be submitted to the Center for Scientific Review (CSR) at the NIH**
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Grants

Small Business Innovation Research Program

- Requirement for Federal agencies with extramural budgets over \$100 million
 - Set-aside program for small businesses to engage in federal R&D
 - Potential for commercialization and public benefit using an annual set-aside of 2.5 %
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Grants

Small Business Innovation Program (Cont'd)

- **Small business is defined as having 500 or fewer employees**
 - **FDA and NIH have SBIR programs**
 - **FY 2004--FDA (500 thousand) and NIH (500 million)**
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Grants

Small Business Technology Transfer Program (STTR)

- Requirement for Federal agencies with extramural budgets over \$1 billion to administer STTR programs
 - Using an annual set-aside of 0.3 % for small businesses with 500 or fewer employees
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Grants

STTR Program (Cont'd)

- **Objective similar to SBIR**
 - **Unique feature is the requirement for the small business applicant organization to formally collaborate with a research institution**
 - **At least 40 % of the STTR research project is to be conducted by the small business and at least 30 % of the work is to be conducted by the research institution**
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Grants

SBIR and STTR Program Structure

- Phase I: Establishes technical merit and feasibility of the proposed R/R&D efforts
 - Phase II: Continues R&D efforts initiated in phase I with award of additional support
 - Phase III: Pursue with non-SBIR and STTR funds the commercialization of objectives resulting from phase I/II R/R&D activities
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GRANTS

SBIR and STTR Program Structure

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SBIR/STTR Conference

**Annual NIH
SBIR/STTR conference**

**A two-day annual conference
for
small businesses conducting innovative
health-related research**

**The Natcher Conference Center
National Institutes of Health
Bethesda, MD**





Grants and Funding Opportunities at NIH

- **Grants**
- **Research Contracts**
- **NIH Guide for Grants and Contracts**
- **Research Training Opportunities**

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Exclusivity

Exclusivity generally bars FDA approval or acceptance of certain applications for competing drugs.

Exclusivity runs from date of approval. Concurrent with protection (if any), except for pediatric exclusivity which attaches to an existing exclusivity or patent period.



Hatch/Waxman 5 Year Exclusivity

- Purpose was to encourage research and development of new drugs
- 5-year period of exclusivity is granted to new drug products containing chemical entities never previously approved by FDA either alone or in combination
- No 505(b)(2) application or ANDA may be submitted during the 5-year exclusivity period
- Full new drug applications under 505(b)(1) and 505(b)(2) can receive the 5 years of exclusivity

Hatch/Waxman 3 Year Exclusivity

- A 3-year period of exclusivity is granted for a change in an approved drug product.
- Approval requires new clinical investigations (other than bioavailability studies)
- Examples of the changes in an approved drug product that affect its active ingredient(s) are new indication, strength, dosage form, route of administration
- For 3 years FDA may not approve an ANDA (505)(j) or 505(b)(2) for the protected change

Pediatric Exclusivity

- **Six months exclusivity as an incentive to sponsors to conduct more studies of the use of drug in pediatric populations**
- **Attaches to the END of all existing marketing exclusivity and patent periods. Hatch/Waxman-exclusivity, orphan drug exclusivity, and patent periods run concurrently**
- **Only drug products subject to section 505 of the Food, Drug and Cosmetic Act are eligible for pediatric exclusivity**

Generic Drug Exclusivity (180-day Exclusivity)

- **180 days of exclusivity for first ANDA applicant to challenge listed patent**
- **The agency has proposed new regulations in the implementation of the 180-day exclusivity. Have not been finalized**

Patent Term Extension

- **Up to 5 years of patent extension for creating innovative products that benefit the public. Includes most products regulated by FDA including drugs and biologic**
- **Must be the first commercial marketing or use of the product under the provision of the law which such regulatory review occurred**
- **FDA assists patent trademark office (PTO) in determining a products eligibility for patent extension**
- **PTO is responsible for determining the period of patent extension**

Other Federal Assistance

- **Small Business Administration federal grant resources**
- **www.grants.gov**
- **The Catalog of Federal Domestic Assistance**
- **www.gsa.gov/fdac/**
- **NIAID Biodefense Research**
- **www.niaid.nih.gov**

Small Business Assistance Website

www.fda.gov/cder/about/smallbiz/default.htm

- [Information for clinical investigators](#)
- [New drug development and review process](#)
- [Orphan drug program](#)
- [Generic drug review process](#)
- [Over-the-counter drug product review process](#)
- [Post drug-approval activities](#)
- [Drug registration and listing](#)
- [Organization, contact, and meeting information](#)
- [FDA related laws, regulations, and guidances](#)
- [Economic assistance and incentives for drug development](#)
- [Economic assistance, pre-approval](#)
- [Economic incentives post-approval](#)
- [Economic assistance pre-approval and post-approval](#)

Small Business Assistance ListServ

- **What's new for small pharmaceutical businesses:**
- **Receive email notification of new information (federal register notices, guidances, workshops, etc.)**
- **Small Business Assistance Website provides the link**
- **Complete the listserv form.**